THE STATUS OF QUALITY OF LIFE DATA IN HEALTH TECHNOLOGY ASSESSMENTS: EVIDENCE FROM PUBLISHED NICE APPRAISALS

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The requirements for health technology assessments conducted for the National Institute for Clinical Effectiveness (NICE) are similar to those deemed applicable by other groups (for example the Washington Panel). Benefits are represented in terms of quality-adjusted life years (QALYs) where the quality adjustment is based on the utility weights of the relevant reference population. A single index, utility-weighted scoring function that captures the preferences of the general population is problematic. Access to such a weighting system is typically mediated through quality of life (QoL) data that capture the benefits of therapy. Where QoL data do not meet these requirements then alternative methods have to be adopted.

OBJECTIVES: To assess the status of QoL data in the published NICE record and to review the procedures used to estimate utilities based on those data.

METHODS: The 31 assessment reports published by NICE in the period 1999–2001 were systematically reviewed. Particular attention was paid to information provided about QoL measures incorporated in the studies referenced by the appraisal. Of special note was the type of measure (i.e. index or profile), its weighting system and the source of reference values.

RESULTS: QoL data was reported with varying completeness. This contrasts with the data abstraction covering other aspects of the appraisal. Methods used to weight QoL data ranged from the deliberation of expert panels to the use of standardised QoL measures with utility weights elicited from the general population. Evidence of subjective assessment of QALY estimates included their being “based on reasonable assumptions using well-accepted measures”.

CONCLUSIONS: Evidence of the variability in the quality of QoL data and their use in QALY calculations indicates the need for clearer guidance and rigour in reporting and analysis. Findings impact on all parties to this form of technology assessment.

FEATURES OF GOMPERTZ FUNCTIONS IN MODELING MORTALITY RATES

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OBJECTIVES: Despite their surprisingly good fit of current mortality data, Gompertz-functions are rather seldom used in outcome research. The main objective is to show some interesting methodological features of the Gompertz function encouraging a more wide-spread use in modeling mortality rates beyond the time frame of clinical trials.

METHODS: Current mortality data of the Statistical Yearbook of Germany are closely fitted by Gompertz functions \( R_x = R_o \exp(bx) \) where \( x = \text{age} \) which involves estimating parameters \( b \) und \( R_0 \) by non-linear iterative regression. Several authors have observed that in a series of Gompertz-functions from the same underlying population (i.e. life tables from different years, different subgroups, different regions etc.) the two parameters are closely related as: \( \ln R_0 = \alpha b + \beta \), where \( \alpha < 0, \beta \in \mathbb{R} \). Based on this relation, it is shown how to determine Gompertz functions for specific study cohorts by specifying a standardised mortality ratio (SMR) at a certain age. In practical applications this SMR can be estimated from clinical trials data.

RESULTS: All Gompertz-functions satisfying the relation \( \ln R_0 = \alpha b + \beta \) intersect at \((-\alpha, \exp(\beta))\). If the SMR is fixed at an age of \( x' \) then a Gompertz-function satisfying this condition is given by \( b_2 = \ln(\text{SMR})/(x' + \alpha) + b_1 \), and \( R_0 = \exp(\alpha b_2) \exp(\beta) \), where \( (b_2, R_0) \) is the pair of parameters of the new Gompertz-function, and the pair \( (b_1, R_{10}) \) belongs to the baseline Gompertz-function. Furthermore it holds that the hazard ratio at any age is given by the ratio \( (R_{10}/R_{10})^{\alpha b_2} \).

CONCLUSIONS: The more frequent use of Gompertz-Functions in the area of modeling is motivated by the good fit of mortality data in the general population. Furthermore, this analysis shows how these results can be extended to specific subgroups of patients, when clinical trials data only provide little mortality data. The analysis of high-risk groups in disease prevention seems a sensible context for applying these results.

A UTILITY-MAXIMISATION MODEL OF CHOICE BETWEEN MEDICAL INTERVENTIONS INVOLVING RISK

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OBJECTIVES: To develop a utility-maximisation model of choice between alternative treatments of specific disorders on the basis of microeconomic consumption theory. The model is to take into account patients’ preferences for clinical procedures, possible outcomes and risks involved in alternative treatments.

METHODS: The optimisation problem facing a patient is presented by geometrical and algebraic analysis using an ordinal von Neumann Morgenstern (vNM) utility function in a simple example involving two treatment alternatives, T1 and T2. As the treatments are perfect substitutes, the ratio of marginal utilities \( (dT2/dT1) \) which determines the slope of a patient’s indifference curves is constant and can be identified by means of a modified standard gamble (MSG). By including relative prices of
interventions, optimal allocation decisions are derived, hypothetically assuming a market context.

RESULTS: vNM utility functions are developed for the alternative treatments and a "no treatment" option (T0) on the basis of outcome and probability data from clinical research. Utilities of procedures and outcomes can be identified empirically. This requires a preference ordering of treatment alternatives and MSG questions relating to hypothetical treatments. The number of MSG questions and hypothetical treatments (n-2) depends on the number of vNM variables (n). This procedure identifies the ratio of marginal utilities of T1 and T2, dT2/dT1. The inclusion of relative prices of T1 and T2, pT1/pT2, into the model allows the prediction of utility-based treatment choices and the application of the model in resource allocation.

CONCLUSIONS: The main advantages of this model are its ability to predict utility-based decisions incorporating risk preferences and to avoid the need for methodologically delicate techniques such as sensitivity analysis and discounting. The practical benefits of this approach remain to be determined within empirical evaluations.

PATIENT-GENERATED OUTCOMES: FAD OR HERE TO STAY? Patel KK, Veenstra DL University of Washington, Seattle, WA, USA

Patient-generated outcomes attempt to capture the individualistic nature of quality of life. Although this is an attractive concept, a critical review of these instruments is needed to assess their applicability in a clinical trial setting.

OBJECTIVE: To provide a critical review of four patient-generated quality of life instruments: Patient-Generated Index (PGI), Schedule for the Evaluation of Individual Quality of Life (SEIQoL), Repertory Grid, and Asthma Quality of Life Questionnaire (AQLQ).

METHODS: We conducted a systematic literature review of available computerized databases, the Quality of Life Research Journal and consulted experts in the field. We abstracted data from the studies and constructed a matrix comparing the four instruments based on their psychometric properties and current use in quality of life research.

RESULTS: The PGI and SEIQoL have been shown to be reliable and valid in several different patient populations and disease states; however, neither have been used in a clinical trial. The SEIQoL-DW, in addition, has been shown to be practical and acceptable to patients. The Repertory Grid has been shown to be reliable, valid and practical, but has only been used in one observational study. The AQLQ is a disease-specific instrument that is only partially patient-generated. It has not only shown to be reliable, valid and practical, but has also been used in clinical trials as a sole measure of quality of life.

CONCLUSION: Patient-generated outcomes may not be generally useful in a clinical trial setting. However, hybrid instruments, such as the AQLQ, may be applicable in a clinical trial setting. The primary role of patient-generated outcome measures is as an adjunct measure or to guide individual patient treatment decisions.